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TITLE:

Long term expression of recombinant human

factor IX mediated by myoblasts in

mice

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The exptl. gene therapy for hemophilia B by using C2C12 cells and C3H

mice as models was developed. The retroviral vector contg. MCK enhancer and hCMV promoter was constructed and was used to transfect PA317 cells. Then the viral particles were used to

transfect C2C12 cells. C2C12 cells expressing human factor IX (hFIX) protein in vivo was pos. correlated

with the no. of injected cells, and the in vitro expression level of

cell clones. The expression of hFIX could be enhanced and prolonged by immunosuppressive agents. Allogeneic myoblasts transplantation led to immunorejection, but repeated injections at longer internal could improve the survival rate of transplanted cells.

FILE 'MEDLINE, CAPLUS, BIOSIS' ENTERED AT 10:31:01 ON 10 DEC 2001 10623 S FACTOR IX OR BILIRUBIN UDP GLUCURONOSYLTRANSFERASE L1236 S L1 AND VECTOR AND PROMOTER L2 10153 S FACTOR IX L3 L4236 S L1 AND VECTOR AND PROMOTER L5 1 S L4 AND SERTOLI L6 4 S L4 AND (TRANSFORM OR TRANSFECT) 4 DUP REMOVE L6 (0 DUPLICATES REMOVED) L7 rs14014 S SERTOLI CELLS 27 S L8 AND (TRANSFECT OR TRANSFORM) L9 15 DUP REMOVE L9 (12 DUPLICATES REMOVED)

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| L1 | 10623 | S FACTOR IX OR BILIRUBIN UDP GLUCURONOSYLTRANSFERASE |
| L2 | 236 | S L1 AND VECTOR AND PROMOTER |
| L3 | 10153 | S FACTOR IX |
| L4 | 236 | S L1 AND VECTOR AND PROMOTER |
| L5. | 1 | S L4 AND SERTOLI |
| L6 | 4 | S L4 AND (TRANSFORM OR TRANSFECT) |
| L7 | 4 | DUP REMOVE L6 (0 DUPLICATES REMOVED) |
| L8 | 14014 | S SERTOLI CELLS |
| L9 | 27 | S L8 AND (TRANSFECT OR TRANSFORM) |
| L10 | 15 | DUP REMOVE L9 (12 DUPLICATES REMOVED) |